

FIG. 1

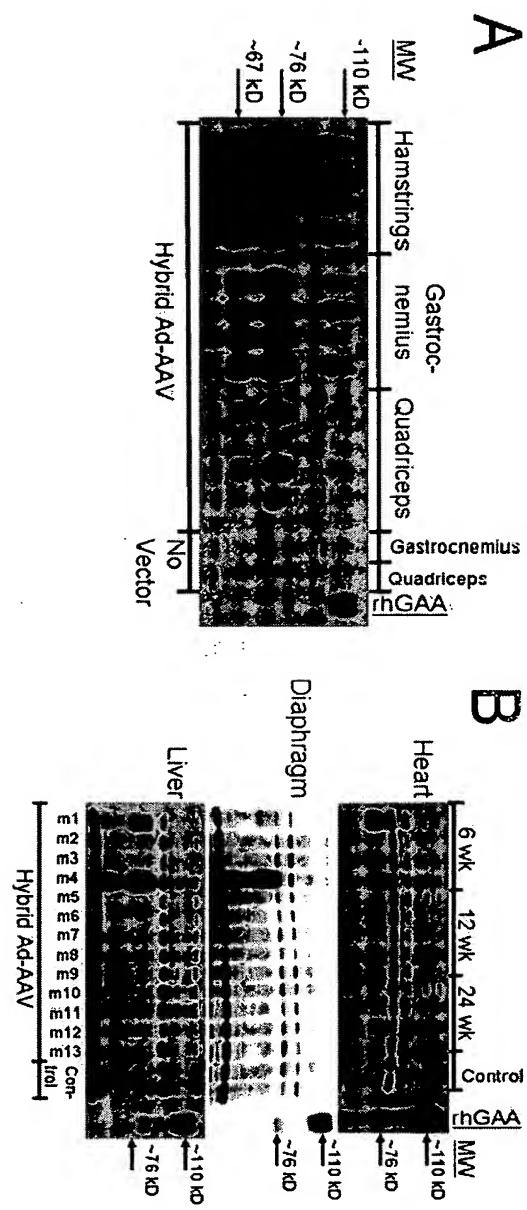


FIG. 2

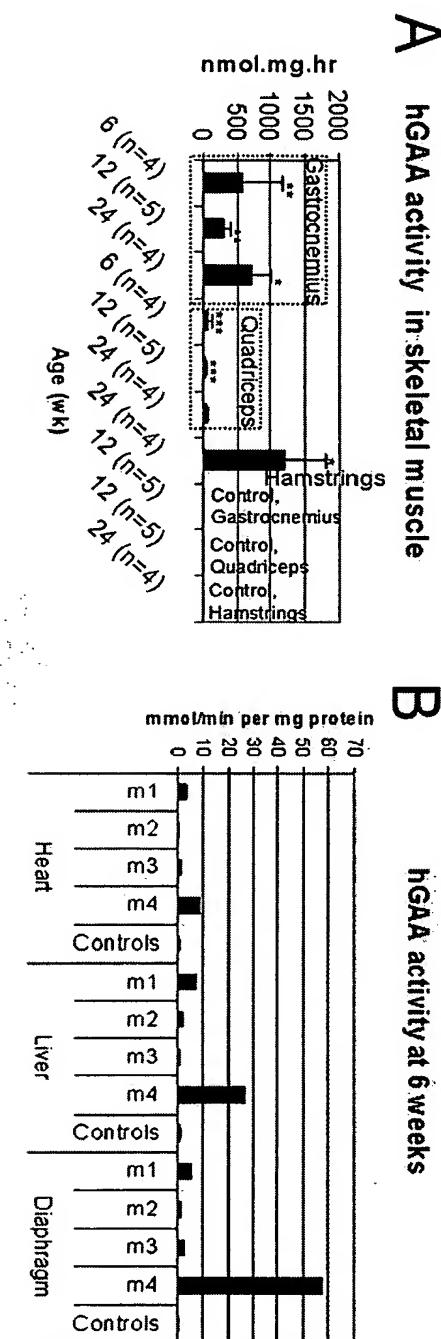


FIG. 3

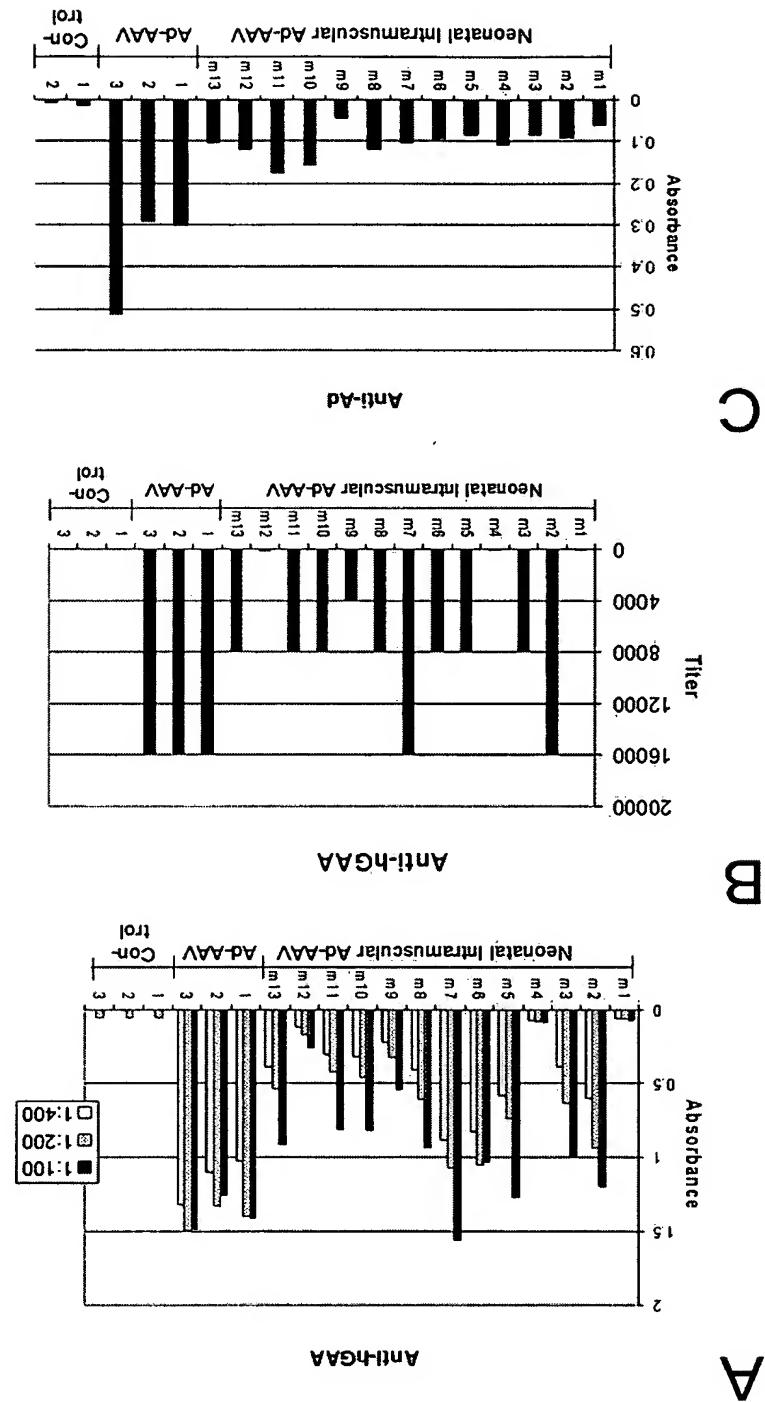


FIG. 4

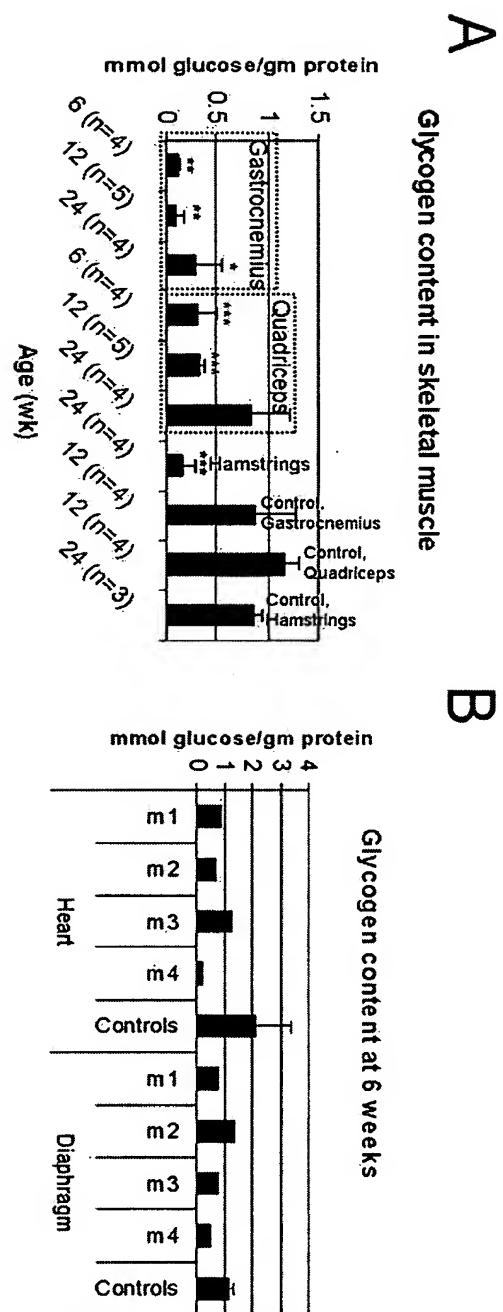
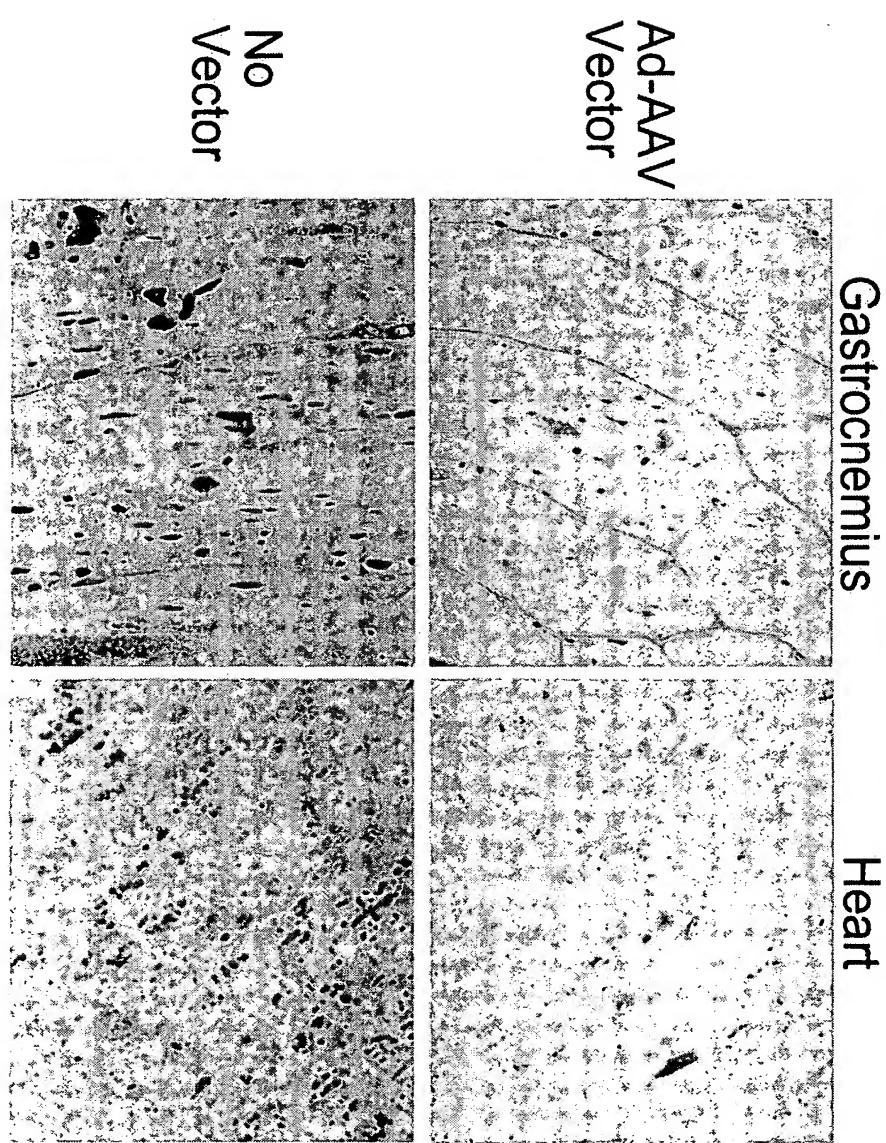
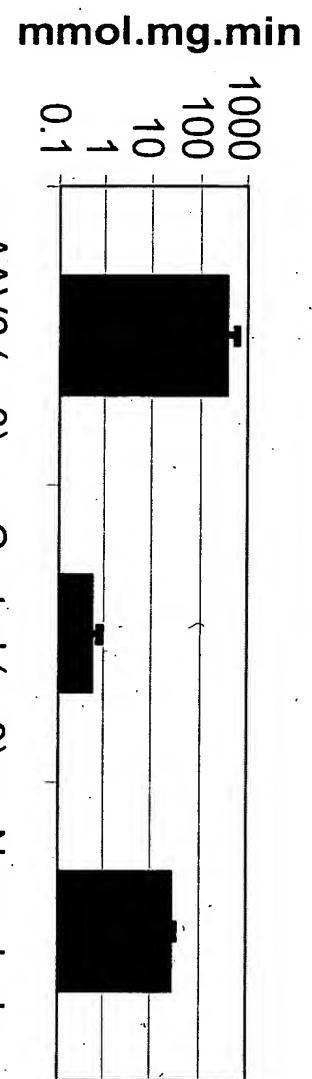


FIG. 5



**hGAA with an AAV6 vector in mouse
muscle**



**Reduced glycogen content following AAV6
vector administration**

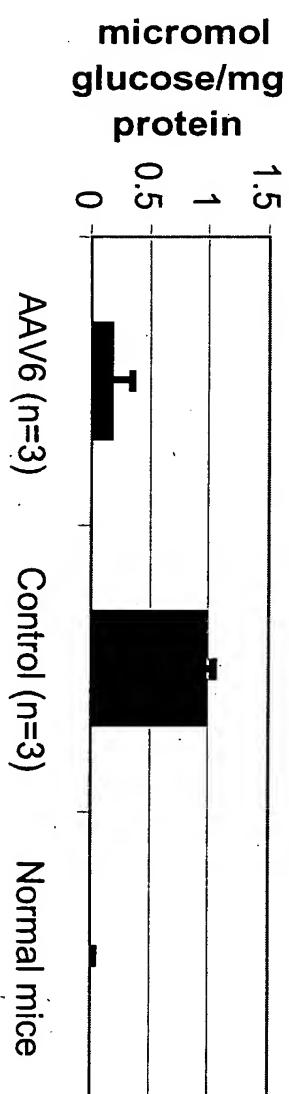


FIG. 6

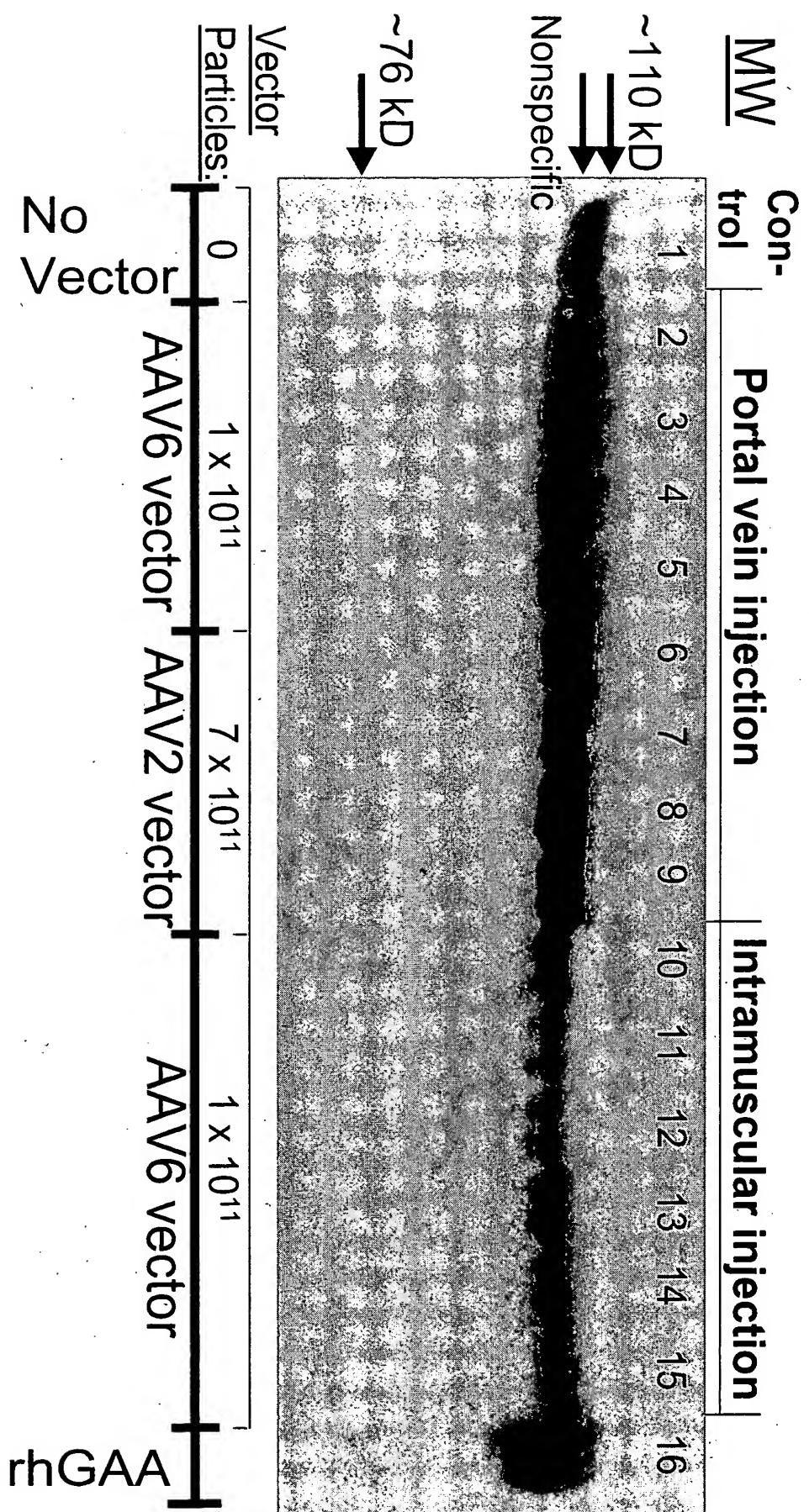


FIG. 7

Figure 8

The full-length human GAA cDNA sequence (3846 bp, Genebank number: NM_000152).

1 *gcccgtgcgc gggaggccgc gtcacgtgac ccaccgcggc cccgcggc gacgagctcc*
61 *cgccggtcac gtgacccgc tctgcgcgc cccgggcacg accccggagt ctccgcggc*
121 *ggccaggcg cgctgcgcg gaggtgagcc gggccggggc tgcggggctt ccctgagcgc*
181 *gggcgggtc gggtggcgg tcggctccc gcgcggcctt cttagttggg aaagctgagg*
241 *ttgtcgccgg ggccgcgggt ggaggcggg gatgaggcag caggtaggac agtgaccccg*
301 *gtgacgcgaa ggaccccgcc cacccttagg ttctcctcgt ccgcggcttg ttcaagcgagg*
361 *gaggctctgg gcctgcccga gctgacgggg aaactgaggc acggagcggg cctgtaggag*
421 *ctg tccaggc catctccaac catgggagt aggaccccgc cctgctccca ccggctccctg*
481 *gccgtctcg ccctctgtc ctggcaacc gctgcactcc tggggcacat cctactccat*
541 *gattccctgc tggttccccg agagctgagt ggctcctccc cagtcctgga ggagactcac*
601 *ccagctcacc agcagggagc cagcagacca gggcccccggg atgcccaggg acaccccgcc*
661 *cgtcccaagag cagtccac acagtgcgc acgtggccat cggccgcgtt cgattgcgc*
721 *cctgacaagg ccatcacccca ggaacagtgc gaggcccgcg gtcgtgcta catccctgca*
781 *aagcaggggc tgcagggagc ccagatgggg cagccctgtt gtccttccc acccagctac*
841 *cccagctaca agctggagaa cctgagctcc tctgaaatgg gtcacacggc caccctgacc*
901 *cgtaccaccc ccaccctt ccccaaggac atcctgaccc tgccgctgga cgtgtatgt*
961 *gagactgaga accgcctcca cttcacgatc aaagatccag ctaacaggcg ctacgagggt*
1021 *cccttggaga ccccgctgt ccacagccgg gcacccgtt cactctacag cgtggagttc*
1081 *tccgaggagc cctcgggtt gatcgtgcac cggcagctgg acggccgcgt gtcgtgaac*
1141 *acgacggtgg cgcctgtt cttgcggac cagttccac agctgtccac ctgcgtccccc*
1201 *tcgcagata tcacaggcct cgccgagcac ctcaatcccc tgcgttcac caccagctgg*
1261 *accaggatca ccctgtggaa ccgggacatt ggcggccacgc ccgggtgcgaa cctctacggg*
1321 *tctaccctt tctacccggc gtcggaggac ggcgggtcg cacacgggtt gttctgtcta*
1381 *aacagcaatg ccatggatgt gtcctgcag ccgcggccctg cccttagctg gaggtcgaca*
1441 *ggtggatcc tggatgtcta catcttctg gcccagagc ccaagagcgt ggtgcagcag*
1501 *tacctggacg ttgtggata cccgttcatg ccgcataact ggggcctggg ctcccacctg*
1561 *tgccgtgg gctactcctc caccgcatac acccgccagg tggggagaa catgaccagg*
1621 *gcccacitcc ccctggacgt ccaatggaaac gacctggact acatggactc ccggagggac*
1681 *ttcacgttca acaaggatgg ctccggac tccccggca ttgtgcagga gtcgcaccag*
1741 *ggcggccggc gtcacatgt gatcgtggat ctcgcatac gcagctggg ccctgcccgg*
1801 *agctacaggc ctcacgcga gggctcggtt aggggggtt tcacatccaa cgagaccggc*
1861 *cagccgtga ttggaaaggat atggccggg tccactgcct tccccactt caccaccc*
1921 *acagccctgg ctcggggaa ggacatgggt gtcgtgtcc atgaccaggt gcccctcgac*
1981 *ggcatgtgga ttgacatgaa cgacccatca aacttcatac gaggctctga ggacggctgc*
2041 *cccaacaatg agctggagaa cccaccctac gtgcctggg tgggtgggg gaccctccag*
2101 *gcccgcacca tctgtccctc cagccaccag ttctctccaa cacactacaa cctgcacaac*
2161 *ctctacggcc tgacccaaacg catgcctcc cacagggcgc tggtaaggc tcgggggaca*
2221 *cgcccaattt gatcctcccg ctcgcaccc gtcggccacg gccgatacgc cggccactgg*
2281 *acgggggacg tggagcttc ctggagcag ctcgcctt ccgtgccaga aatccctgcag*

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2341 tttaacctgc tgggggtgcc tctggcggg gccgacgtct gcggcttcct gggcaacacc
2401 tcagaggagc tgggtgtcgctggacccag ctgggggcct tctacccctt catgcggAAC
2461 cacaacagcc tgctcagtct gcccaggag ccgtacagct tcagcgagcc ggcccagcag
2521 gccatgagga aggccctcac cctgcgtac gcactctcc cccacctta cacactgttc
2581 caccaggccc acgtcgcggg ggagaccgtg gcccggccccc tcttcgtga gttccccaaag
2641 gactctagca cctggactgt ggaccaccag ctccgtggg gggaggccct gtcatcacc
2701 ccagtgtcc agggcgggaa ggccgaagtg actggctact tcccttggg cacatggta
2761 gacctgcaga cggtgccaaat agaggccctt ggcagccctc caccggcacc tgcagctccc
2821 cgtgagccag ccatccacag cgagggccag tgggtgacgc tgccggccccc cctggacacc
2881 atcaacgtcc acctccgggc tgggtacatc atccccctgc agggccctgg ctcacaacc
2941 acagagtccc gccagcagcc catggccctg gctgtggccc tgaccaaggg tggagaggcc
3001 cgagggggagc tgggtgtggga cgatggagag agcctggaaag tgcgtggagcg aggggcctac
3061 acacagggtca tcttcctggc caggaataac acgtatgtga atgagctggt acgtgtgacc
3121 agtgaggaggag ctggcgtgc a gctgcagaag gtgactgtcc tggcgtggc cacggcggcc
3181 cagcaggtcc tctccaacgg tgcctgttc tccaacttca cctacagccc cgacaccaag
3241 gtcctggaca tctgtgtctc gctgtgtatggagagcagt ttctcgtagc ctgggttt**tag**
3301 cggggcggag tgggttagtc tctccagagg gaggctggg ccccaggaa gcagagccctg
3361 tggcgggca gcagctgtgt gggggccctgg ggggtt *catg tgcacccctgg agctggcac*
3421 *taaccattcc aagccggccgc atcgcttgc tccacccctt gggccggggc tctggccccc*
3481 *aacgtgtcta ggagagctt ctccctagat cgcactgtgg gcccggccct ggagggtgc*
3541 *tctgtgttaa taagatgtaa aggtttggcc tccacccctt tggcggcat ggggttagta*
3601 *ttagccaccc ccctccatct gtcccccagca cggagaagg ggggtgcag gtggagggtgt*
3661 *ggggatgtca cctgagctcc tgcgtgcgc ctgcgtctt gcccacgc gaccgcitcc*
3721 *cggctggccca gagggtggta tgcctggccgg tccctggagca agccctggaa ctcaggaaaa*
3781 *ttcacaggac ttgggagatt ctaaaatc tta agtgcatttt aataaaa aggggcattt*
3841 ggaatc

Figure 9

5' and 3' deleted GAA

1 409 410 g cctgttaggag
421 ctg tccaggc catccaaac **catggagtg** aggcacccgc cctgctccca ccggctcctg
481 gccgtctcg ccctcggtc ctggcaacc gctgcactcc tggggcacat cctactccat
541 gattcctgc tggcccccg agagctgagt ggctcctccc cagtcctgga ggagactcac
601 ccagctcacc agcagggagc cagcagacca gggccccggg atgcccaggc acaccccgcc
661 cgtcccaagag cagtccccac acagtgcgac gtcccccca acagccgctt cgattgcgc
721 cctgacaagg ccatcaccca ggaacagtgc gaggccccgc gctgctgcta catccctgca
781 aagcaggggc tgcagggagc ccagatgggg cagccctggt gcttcttccc acccagctac
841 cccagctaca agctggagaa cctgagctcc tctgaaatgg gctacacggc caccctgacc
901 cgtaccaccc ccacccctt ccccaaggac atcctgaccc tgcggctgga cgtgatgatg
961 gagactgaga accgcctca cttcacgatc aaagatccag ctaacaggcg ctacgaggtg
1021 cccttggaga ccccgctgt ccacagccgg gcacccgtccc cactctacag cgtggagttc
1081 tccgaggagc cttcggggt gatcggtcac cggcagctgg acggccgcgt gctgctgaac
1141 acgacgggtgg cgccctgtt ctttgcggac cagttccctc agctgtccac ctcgctgccc
1201 tcgcagata tcacaggcct cgccgagcac ctcaatcccc ttagtgcac caccagctgg
1261 accaggatca cccttggaa ccgggacctt ggcgcacgc ccgggtcgaa cctctacggg
1321 ttcacccctt ttcacccggc gctggaggac ggcgggtcggt cacacgggggt gttccctgcta
1381 aacagcaatg ccatggatgt gtcctgcag ccgagccctg cccttagctg gaggtcgaca
1441 ggtgggatcc tggatgtcta catcttccgt ggcccgagac ccaagagcgt ggtgcagcag
1501 tacctggacg ttgtggata cccgttcatg cggccataact ggggcctggg cttccacctg
1561 tgccgtggg gctactcctc caccgctatc acccgccagg tggggagaa catgaccagg
1621 gcccacttcc ccctggacgt ccaatggaaac gacctggact acatggactc cgggagggac
1681 ttcacgttca acaaggatgg ctccggac ttccggcca tggtgccatg gtcgcaccag
1741 ggcggccggc gtcacatgtat gatcggtat ctcgtccatca gcagctggg ccctgcccgg
1801 agctacaggc ctcacgacga gggctgtggg aggggggtt tcatcaccaaa cgagaccggc
1861 cagccgctga ttgggaaggt atggcccggtt tccactgcct tcccgactt caccaacccc
1921 acagccctgg cttgggtggg ggacatgggt gctgagttcc atgaccagg gcccctcgac
1981 ggcacatgtggaa ttgacatgaa cggcccttcc aacttcatca gaggctctga ggacggctgc
2041 cccaaatg agctggagaa cccaccctac gtgcctgggg tgggtgggg gaccctccag
2101 gcccacca tctgtgcctc cagccaccag ttctcttca cacactacaa cctgcacaac
2161 ctctacggcc tgaccgaagc catcgctcc cacagggcgc tggtaaggc tggggggaca
2221 cggccatttg tggatcccg ctgcaccctt gtcggccacg gccgatacgc cggccactgg
2281 acgggggacg tggatgtgc ctggagcag ctgcctccct ccgtgccaga aatccgtcag
2341 tttaacctgc tgggggtgcc tctgggtggg gccgacgtct gccggcttccctt gggcaacacc
2401 tcagaggagc tggatgtgc ctggacccag ctgggggcct tctaccctt catgcggaaac
2461 cacaacagcc tgctcgtctt gcccaggag cggatcagct tcagcgagcc gcccagcag

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2521 gccatgagga aggccctcac cctgcgctac gcactcctcc cccacctcta cacactgttc
2581 caccaggccc acgtcgccggg ggagaccgtg gccccggccc tcttcctgga gttccccaag
2641 gactctagca cctgactgt ggaccaccag ctccctgtggg gggaggccct gctcatcacc
2701 ccagtgcctcc agggcgggaa ggccgaagtg actggctact tcccttggg cacatggtac
2761 gacctgcaga cggtgccaaat agaggccctt ggcagcctcc caccccccacc tgcagctccc
2821 cgtgagccag ccatccacag cgagggcag tgggtgacgc tgccggccccc cctggacacc
2881 atcaacgtcc acctccgggc tgggtacatc atccccctgc agggccctgg cctcacaacc
2941 acagagtcctt gccagcagcc catggccctg gctgtggccc tgaccaaggg tggagaggcc
3001 cgaggggagc tggcttggga cgtatggagag agcctggaag tgcggagcg aggggcctac
3061 acacagggtca tcttcctggc caggaataac acgtatgtga atgagctggt acgtgtgacc
3121 agtgagggag ctggcctgca gctgcagaag gtgactgtcc tggcgtggc cacggccccc
3181 cagcagggtcc tctccaacgg tggccctgtc tccaacttca cctacagccc cgacaccaag
3241 gtcctggaca tctgtgtctc gctgttgatg ggagagcagt ttctcgtag ctggtgtag
3301 ccggggggag tggctttagtc tctccagagg gaggctgggtt cccaggaa gcagagccctg
3361 tgtgcgggca gcagctgtgt gcggggcctgg gggttg 3397.....3807
3808 tta agtcaatttataaaa aggggcatttggaaatc

FIG. 10

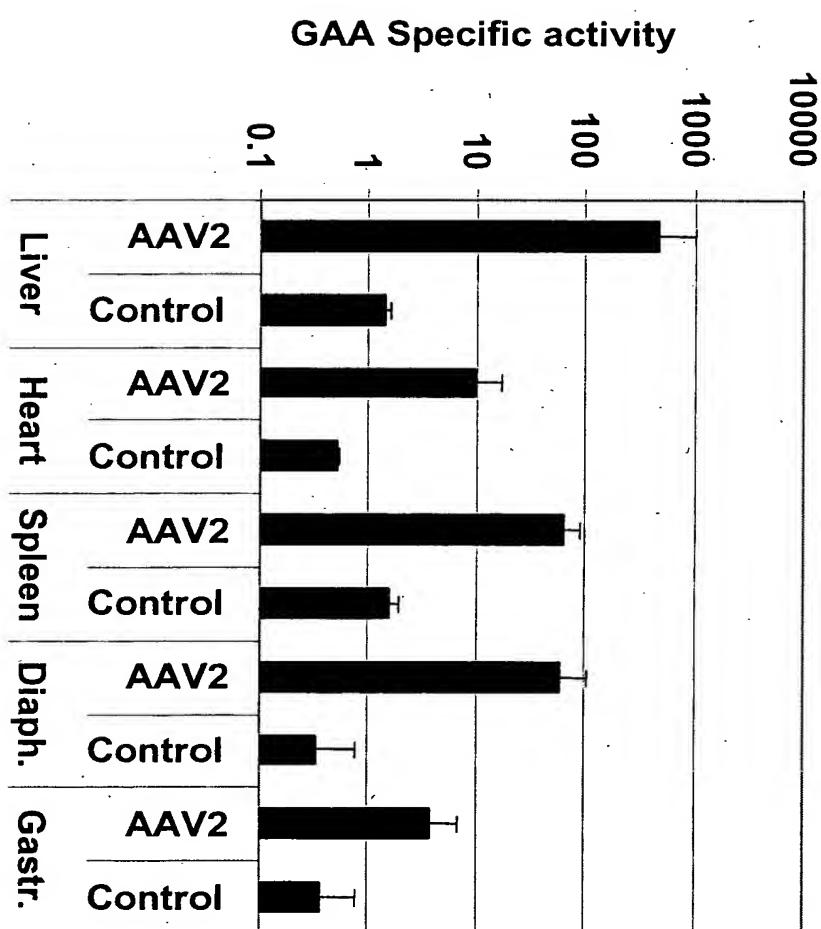


FIG. 11

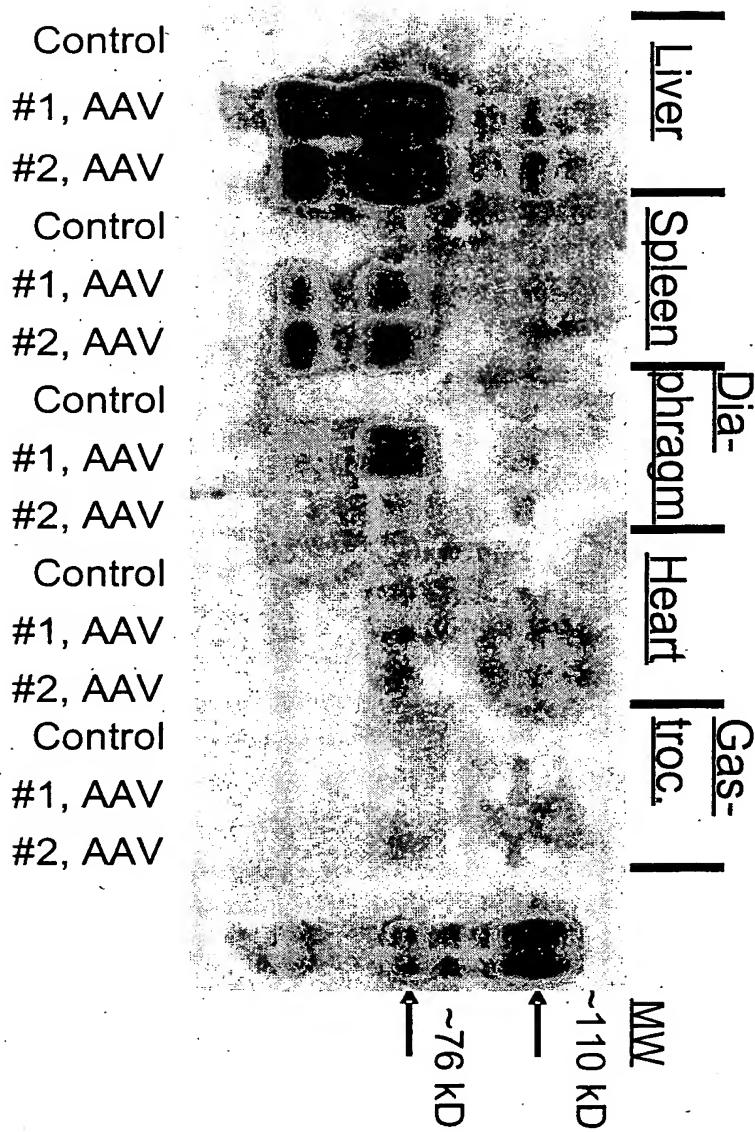


FIG. 12

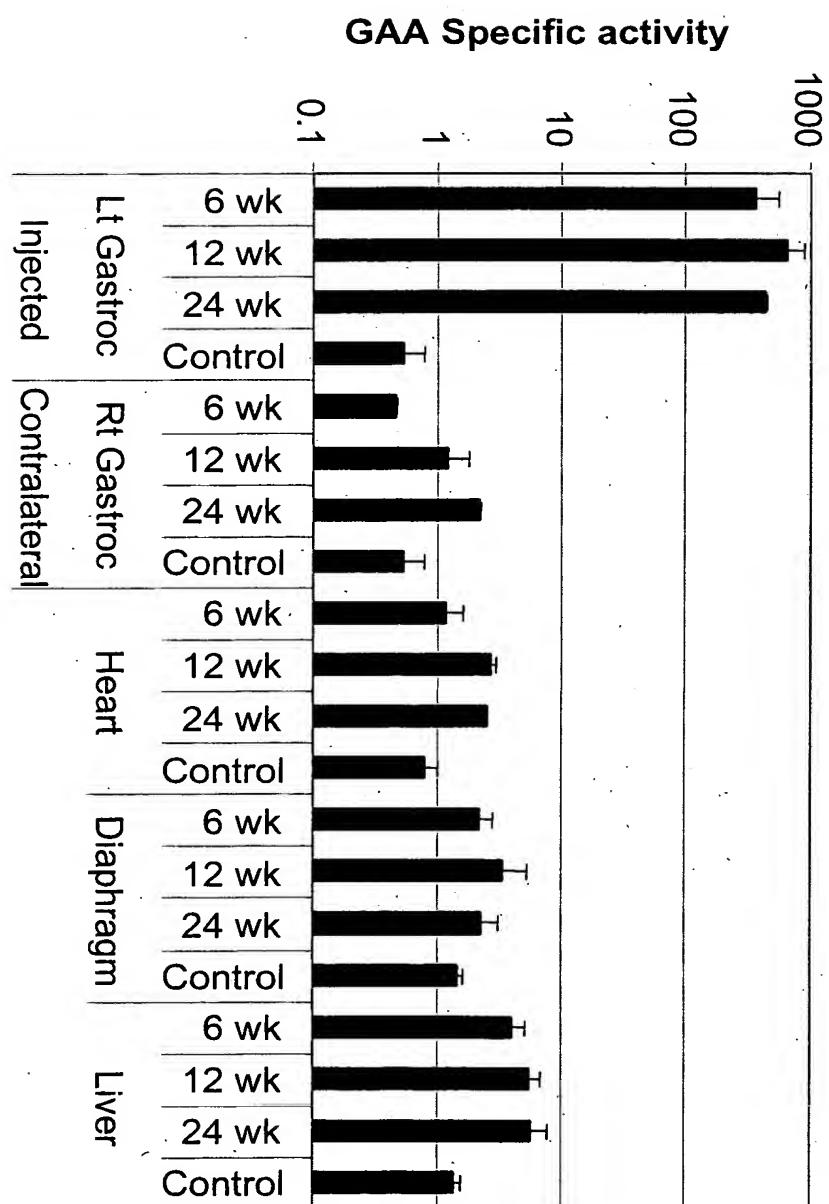
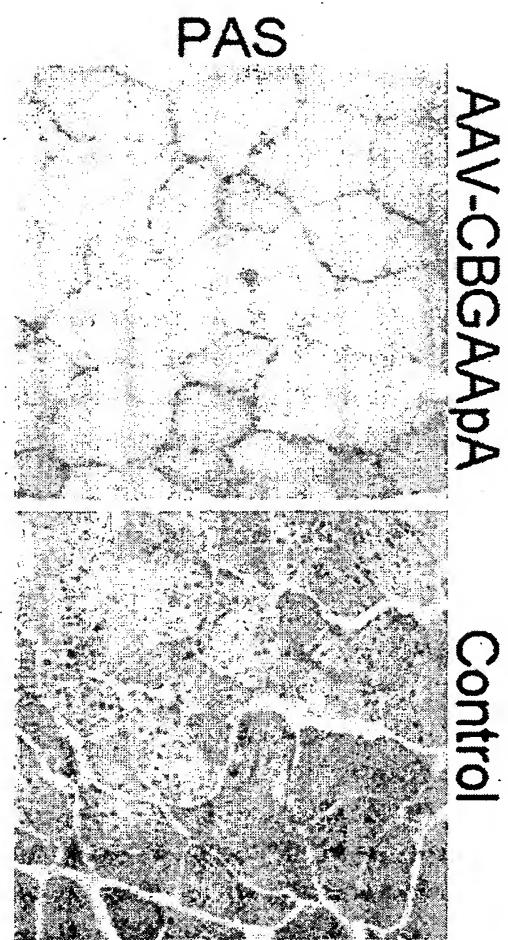
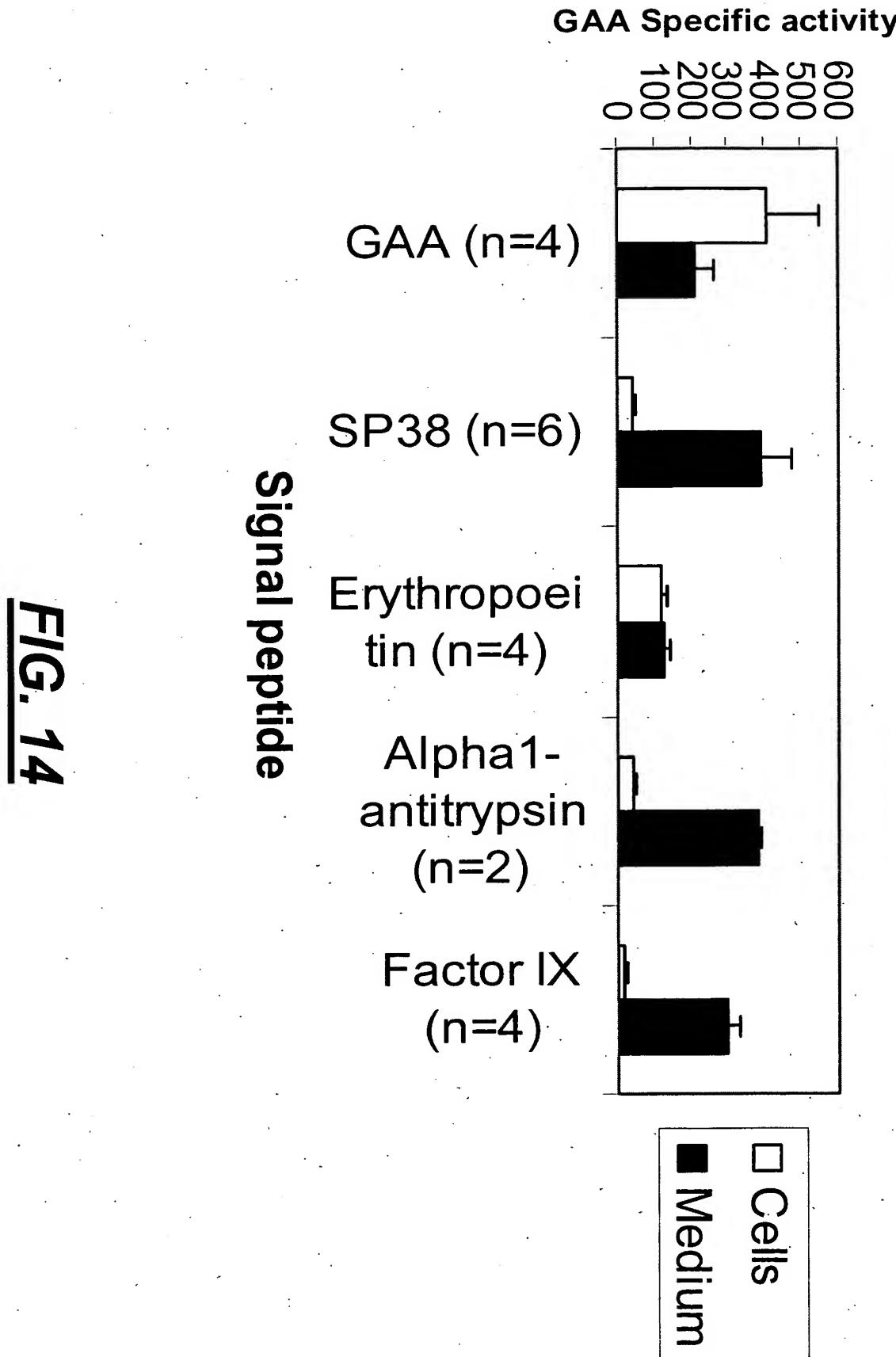


FIG. 13



GAA in transfected 293 cells



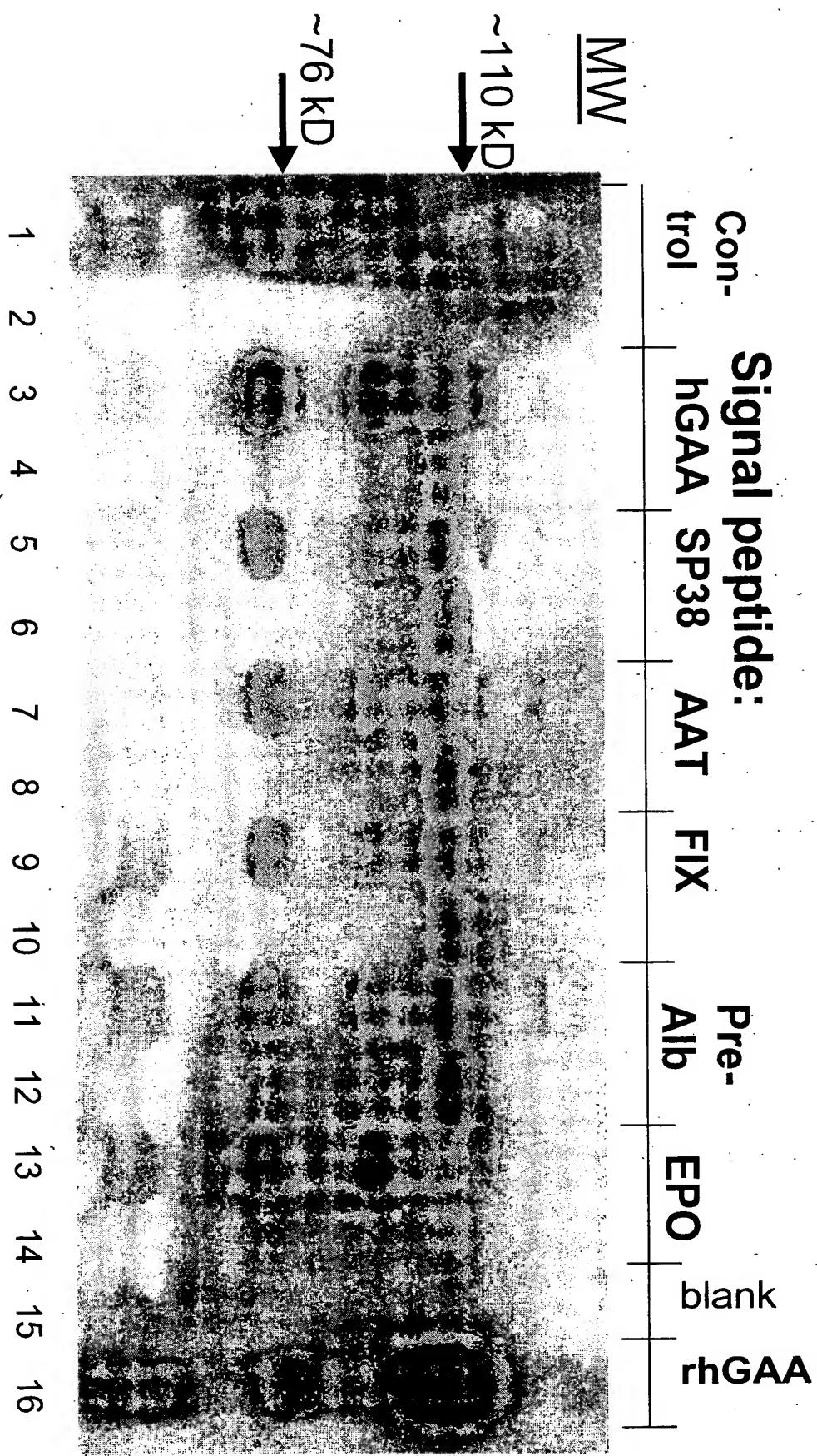


FIG. 15

FIG. 16

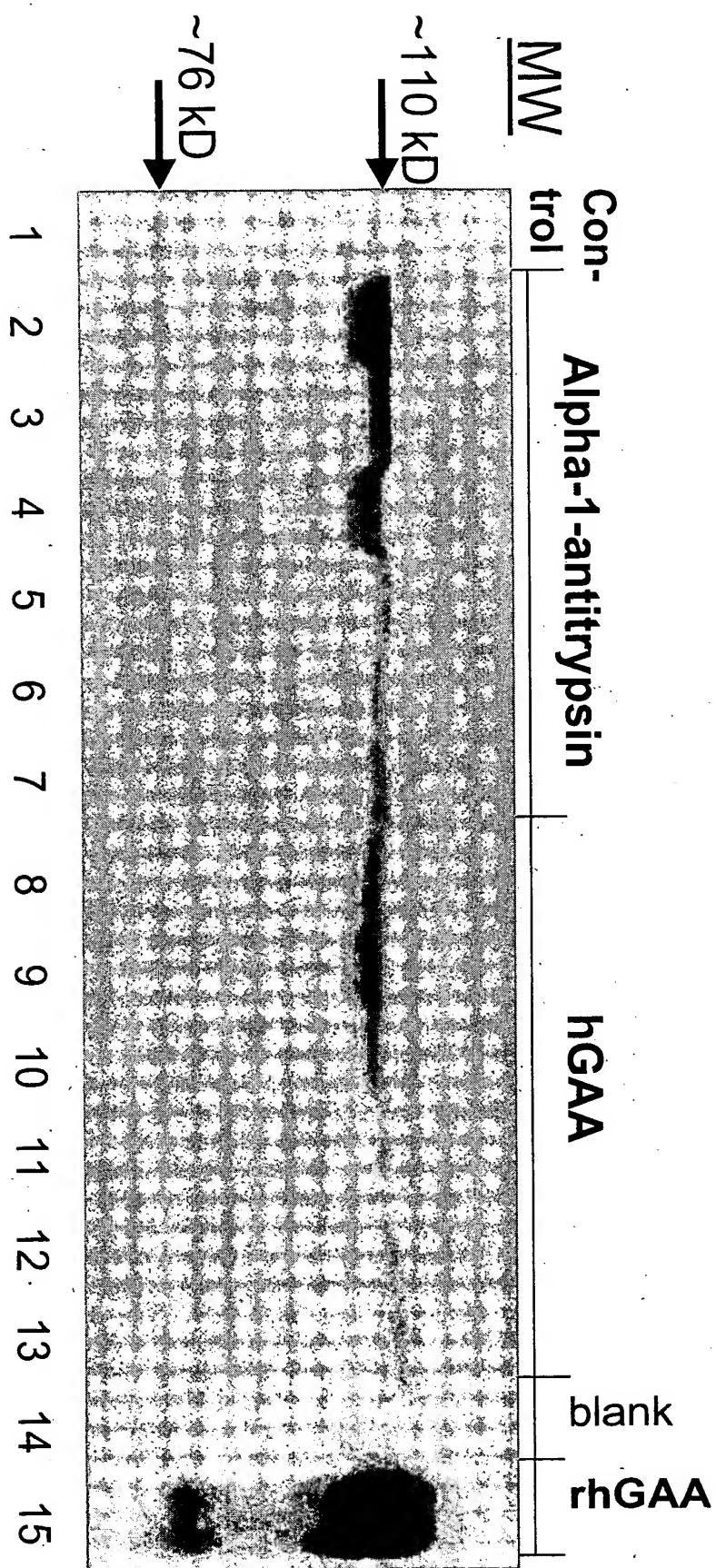


FIG. 17

Human GAA with Alpha-1-antitrypsin signal peptide

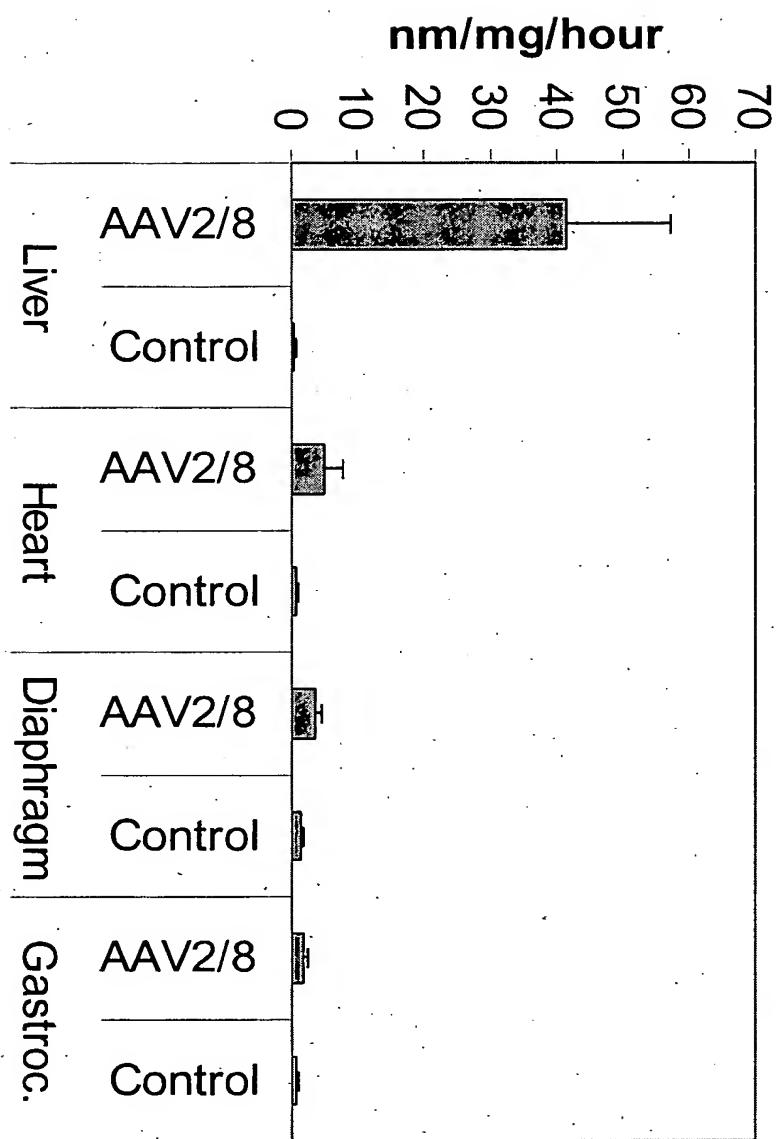


FIG. 18

